Insulin expression and C-peptide in type 1 diabetes subjects implanted with stem cell-derived pancreatic endoderm cells in an encapsulation device.

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Public Summary:
This paper reports preliminary evidence that in 17 people with type 1 diabetes, precursor pancreatic cells loaded into an investigational device implanted underneath the skin (VC-02) were able to survive and express insulin in 63% of units at 3–12 months post-implant. In addition, expression of C-peptide, a biomarker for insulin synthesis and secretion, was demonstrated in 35.3% of subjects as early as 6 months post-implant. The product and implantation procedure were well tolerated. Pluripotent stem cells may be a scalable, renewable alternative to pancreatic islet transplants and could potentially provide a functional cure for type 1 diabetes and thus possibly eliminate the need for insulin injections.

Scientific Abstract:
These preliminary data from an ongoing first-in-human phase 1/2, open-label study provide proof-of-concept that pluripotent stem cell-derived pancreatic endoderm cells (PEC-01) engrafted in type 1 diabetes patients become islet cells releasing insulin in a physiologically regulated fashion. In this study of 17 subjects aged 22-57 with type 1 diabetes, PEC-01 cells were implanted subcutaneously in VC-02 macroencapsulation devices, allowing for direct vascularization of the cells. Engraftment and insulin expression were observed in 63% of VC-02 units explanted from subjects at 3-12 months post-implant. Six of 17 subjects (35.3%) demonstrated positive C-peptide as early as 6 months post-implant. Most reported adverse events were related to surgical implant or explant procedures (27.9%) or to side-effects of immunosuppression (33.7%). Initial data suggest that pluripotent stem cells, which can be propagated to the desired biomass and differentiated into pancreatic islet-like tissue, may offer a scalable, renewable alternative to pancreatic islet transplants.